



4160-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2014-N-0396]

Public Meeting on Patient-Focused Drug Development for Neurologic Manifestations of Inborn Errors of Metabolism

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public meeting; request for comments.

SUMMARY: The Food and Drug Administration (FDA or Agency) is announcing a public meeting and an opportunity for public comment on Patient-Focused Drug Development for neurologic manifestations of inborn errors of metabolism. Patient-Focused Drug Development is part of FDA's performance commitments made as part of the fifth authorization of the Prescription Drug User Fee Act (PDUFA V). The public meeting is intended to allow FDA to obtain patient perspectives on the impact of neurologic manifestations of inborn errors of metabolism on daily life as well as patient views on treatment approaches for neurologic manifestations of inborn errors of metabolism.

DATES: The public meeting will be held on June 10, 2014, from 9 a.m. to 1 p.m. Registration to attend the meeting must be received by May 27, 2014 (see the SUPPLEMENTARY INFORMATION section for instructions).

ADDRESSES: The meeting will be held at the FDA White Oak Campus, 10903 New Hampshire Ave., Building 31 Conference Center, the Great Room (rm. 1503), Silver Spring, MD 20993.

Entrance for the public meeting participants (non-FDA employees) is through Building 1, where routine security checks will be performed. For more information on parking and security

procedures, please refer to

<http://www.fda.gov/AboutFDA/WorkingatFDA/BuildingsandFacilities/WhiteOakCampusInformation/ucm241740.htm>.

Submit either electronic or written comments by August 11, 2014. Submit electronic comments to [www.regulations.gov](http://www.regulations.gov). Submit written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number found in brackets in the heading of this document.

FDA will post the agenda approximately 5 days before the meeting at:

<http://www.fda.gov/Drugs/NewsEvents/ucm387057.htm>.

FOR FURTHER INFORMATION CONTACT: Pujita Vaidya, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 51, rm. 1170, Silver Spring, MD 20993, 301-796-0684, FAX: 301-796-0684, email: [Pujita.Vaidya@fda.hhs.gov](mailto:Pujita.Vaidya@fda.hhs.gov).

#### SUPPLEMENTARY INFORMATION:

##### I. Background on Patient-Focused Drug Development

FDA has selected neurologic manifestations of inborn errors of metabolism as the focus of a public meeting under Patient-Focused Drug Development, an initiative that involves obtaining a better understanding of patients' perspectives on the severity of the disease and the available therapies for the condition. Patient-Focused Drug Development is being conducted to fulfill FDA performance commitments that are part of the authorization of PDUFA V under Title I of the Food and Drug Safety and Innovation Act (FDASIA) (Pub. L. 112-144). The full set of

performance commitments is available on the FDA Web site at

<http://www.fda.gov/downloads/forindustry/userfees/prescriptiondruguserfee/ucm270412.pdf>.

FDA committed to obtain the patient perspective on 20 disease areas during the course of PDUFA V. For each disease area, the Agency will conduct a public meeting to discuss the disease and its impact on patients' daily lives, the types of treatment benefit that matter most to patients, and patients' perspectives on the adequacy of the available therapies. These meetings will include participation of FDA review divisions, the relevant patient communities, and other interested stakeholders.

On April 11, 2013, FDA published a notice (78 FR 08441) in the Federal Register announcing the disease areas for meetings in fiscal years (FYs) 2013-15, the first 3 years of the 5-year PDUFA V timeframe. The Agency used several criteria outlined in the April 11, 2013, notice to develop the list of disease areas. FDA obtained public comment on the Agency's proposed criteria and potential disease areas through a public docket and a public meeting that was convened on October 25, 2012. In selecting the set of disease areas, FDA carefully considered the public comments received and the perspectives of review divisions at FDA. By the end of FY 2015, FDA will initiate a second public process for determining the disease areas for FY 2016-17. More information, including the list of disease areas and a general schedule of meetings, is posted on FDA's Web site at

<http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm326192.htm>.

## II. Public Meeting Information

### A. Purpose and Scope of the Meeting

The purpose of this Patient-Focused Drug Development meeting is to obtain input on the symptoms and other impacts of neurologic manifestations of inborn errors of metabolism that matter most to patients, as well as perspectives on current approaches to treating neurologic

manifestations of inborn errors of metabolism. FDA expects that this information will come directly from patients, caregivers, and patient advocates. Inborn errors of metabolism include a range of genetic disorders in which the body has an enzyme deficiency, which causes buildup of harmful metabolites. Examples of inborn errors of metabolism include phenylketonuria, lysosomal storage disorders, Wilson disease, and many others. Symptoms vary depending on the condition and can be acute or chronic. Neurologic symptoms are common. For most inborn errors of metabolism, a cure does not exist, and treatment focuses on prevention and management of signs and symptoms.

The questions that will be asked of patients and patient stakeholders at the meeting are listed in this section, organized by topic. For each topic, a brief initial patient panel discussion will begin the dialogue and will be followed by a facilitated discussion inviting comments from other patient and patient stakeholder participants. In addition to input generated through this public meeting, FDA is interested in receiving patient input addressing these questions through written comments, which can be submitted to the public docket (see ADDRESSES).

For the purposes of this Patient-Focused Drug Development Meeting, FDA is interested in hearing from patients specifically about the neurologic/neuropsychological aspects of inborn errors of metabolism.

For context, please indicate if you are commenting on behalf of a child or loved one who has an inborn error of metabolism. If you are commenting on behalf of a child or loved one, please answer the following questions as much as possible from the patient's perspective.

#### Topic 1: Disease Signs, Symptoms, and Daily Impacts That Matter Most to Patients

1. Of all the signs or symptoms that you/your child experiences because of the condition, which 1-3 neurologic/neuropsychological signs and/or symptoms have the most significant

impact on your/your child's life? (Examples may include seizures, decreased muscle tone, sensory issues, etc.)

2. Are there specific activities that are important to you/your child but that you/your child cannot do because of these neurologic/neuropsychological signs or symptoms? (Examples of activities may include sleeping through the night, daily hygiene, going up the stairs, etc.)

3. How have your/your child's neurologic/neuropsychological signs or symptoms changed over time?

## Topic 2: Patient Perspectives on Current Approaches to Treating Neurologic Manifestations of Inborn Errors of Metabolism

1. What are you/your child currently doing to help treat the condition or its signs/symptoms? (Examples may include prescription medicines, herbal therapies, acupuncture, over-the-counter products, and other therapies including nondrug therapies such as diet modification.)

How well does this current treatment regimen treat the neurological symptoms of your/your child's disease? For example, how well do the treatments improve your/your child's ability to do specific activities?

2. Assuming there is no complete cure for your/your child's condition, what specific attributes would you look for in an ideal treatment for the condition?

3. The process of informed consent is an important way for researchers to communicate the purpose of a clinical trial and the potential benefits and risks of the trial so that people can make an informed decision about whether to participate. Informed consent also ensures that parents are fully informed and are given opportunities to ask questions about the clinical trial. In addition to informed consent from parents, assent from children may also be needed. Assent is

the term used to describe when a child agrees to be in a clinical trial. Among other considerations, children should be old enough to understand basic facts about the clinical trial in order to provide assent to participate.

In the informed consent process, what are important considerations to take into account in cases when the potential participant is a child? For example, how should the informed consent clearly communicate to the patient the potential benefits and risks of a study?

#### B. Meeting Attendance and Participation

If you wish to attend this meeting, visit <http://iempatientfocused.eventbrite.com>. Please register by May 27, 2014. Those who are unable to attend the meeting in person can register to view a live Webcast of the meeting. You will be asked to indicate in your registration if you plan to attend in person or via the Webcast. Your registration will also contain your complete contact information, including name, title, affiliation, address, email address, and phone number. Seating will be limited, so early registration is recommended. Registration is free and will be on a first-come, first-served basis. However, FDA may limit the number of participants from each organization based on space limitations. Registrants will receive confirmation once they have been accepted. Onsite registration on the day of the meeting will be based on space availability. If you need special accommodations because of disability, please contact Pujita Vaidya (see FOR FURTHER INFORMATION CONTACT) at least 7 days before the meeting.

Patients who are interested in presenting comments as part of the initial panel discussions will be asked to indicate in their registration which topic(s) they wish to address. They will be asked to send a brief summary of responses to the topic questions to [PatientFocused@fda.hhs.gov](mailto:PatientFocused@fda.hhs.gov). Panelists will be notified of their selection a few days after the close of registration on May 27, 2014. FDA will try to accommodate all patients and patient

advocate participants who wish to speak, either through the panel discussion or audience participation; however, the duration of comments may be limited by time constraints.

Comments: Regardless of attendance at the public meeting, you can submit electronic or written responses to the questions pertaining to topics 1 and 2 to the public docket (see ADDRESSES) by August 11, 2014. Received comments may be seen in the Division of Dockets Management between 9 a.m. and 4 p.m., Monday through Friday, and will be posted to the docket at <http://www.regulations.gov>.

Transcripts: As soon as a transcript is available, FDA will post it at <http://www.fda.gov/Drugs/NewsEvents/ucm387057.htm>.

Dated: April 22, 2014.

Leslie Kux,

Assistant Commissioner for Policy.

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